

## Summary of the 2009 NF Conference

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The Children's Tumor Foundation 2009 NF Conference was held in Portland, Oregon June 13-16 attracting a record 280 attendees from around the world. The theme of the meeting was 'New Frontiers', highlighting the remarkable progress that has been made in NF research, the progress toward clinical trials and the new challenges emerging. The 2009 Co-Chairs were **Dr. Kathryn North** (*Children's Hospital at Westmead, Sydney*) and **Dr. Joe Kissil** (*The Wistar Institute*). From the opening session, a full afternoon on NF clinical trials, to the final session, on new therapeutic approaches, the span of the conference was a far cry from just a few years ago when the focus was almost exclusively 'discovery' research and is a testament to how rapidly NF research is progressing towards finding effective drug therapies. For the first time the NF Conference included some parallel sessions on a 'clinical' or 'basic' track offering attendees a choice.

### **Some Highlights Reported:**

**NF Clinical Trials:** Phase II NF1 plexiform tumor Rapamycin trial has concluded enrolment; Phase II NF1 learning disabilities Lovastatin trial now open; pilot Phase IIa dermal neurofibroma ranibizumab trial ongoing; Phase II NF1 MPNST RAD001/bevacizumab trial to open late 2009. In NF2, a Phase Zero trial of lapatinib is underway as well as a Phase I/IIa trial of PTC-299.

**New Mouse Models:** Mouse model of NF1 dermal neurofibromas and NF2 meningiomas were described. In addition a new NF1 tumor mouse model was reported that 'progressively' develops different types of NF1 tumors from benign to malignant. Two mouse models of Schwannomatosis are in development and show early promise.

**NF1 Learning Disabilities:** May be due in part to factors determined during early brain 'wiring'.

**New Pipeline Drugs/Pathways:** Schweinfurthins, NCEs based on natural products, target Rac/Rho, of interest for NF1 malignant tumors. Organometallic molecules targeting PI3K/PAK as NF2 drugs. Dual targeting e.g. of mTORC and Akt in NF2 may be of interest as well as dual targeting in NF1.

**Clinical Diagnosis and Management:** A new NF BioBank and a Schwannomatosis database are in development by the Children's Tumor Foundation. We are learning more about Legius Syndrome a Chr. 15 mutation in *Spred-1* that presents clinically as 'mild' NF1. Could account for a small percentage of those people presenting with what looks like NF1, but with no *Nf1* gene mutation.

### **Publicizing Conference Outcome:**

1. NF Conference blog: <http://ctf.org/blog.html> was updated 'live' during the conference.
2. Starting week of July 6, 2009, the attached 10 page report will be released in 'chapters' at [www.ctf.org](http://www.ctf.org) over the coming weeks. Thereafter will be available in full at [www.ctf.org](http://www.ctf.org) and reproduced on our summer 2009 NF NewsLetter (35,000 distributed).
3. For the first time in 2009 the Foundation has been invited to submit a professional meeting report from the Conference for publication in the *American Journal of Medical Genetics*. This is currently being prepared by the Conference Chairs and Session Chairs.